

(19) World Intellectual Property Organization  
International Bureau



(43) International Publication Date  
25 August 2005 (25.08.2005)

PCT

(10) International Publication Number  
**WO 2005/077022 A3**

(51) International Patent Classification<sup>7</sup>: **A01N 63/00**,  
C07H 21/02, 21/04, C12N 15/00, 15/63

(21) International Application Number:  
PCT/US2005/003917

(22) International Filing Date: 4 February 2005 (04.02.2005)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:  
60/542,098 4 February 2004 (04.02.2004) US  
60/555,422 22 March 2004 (22.03.2004) US

(71) Applicant (for all designated States except US): **THE GENERAL HOSPITAL CORPORATION** [US/US]; 55 Fruit Street, Boston, MA 02114 (US).

(72) Inventor; and

(75) Inventor/Applicant (for US only): **KANG, Jing, X.** [US/US]; 28 Meadowood Road, North Andover, MA 01845 (US).

(74) Agent: **CREWS, Lee, Ph., D.**; Fish & Richardson P.C., 225 Franklin Street, Boston, MA 02110-2804 (US).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM,

AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SM, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

**Published:**

- with international search report
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments

(88) Date of publication of the international search report:  
9 February 2006

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: COMPOSITIONS AND METHODS FOR MODIFYING THE CONTENT OF POLYUNSATURATED FATTY ACIDS IN BIOLOGICAL CELLS

(57) Abstract: The present invention features compositions (e.g., nucleic acids encoding fat-1, optionally and operably linked to a constitutively active or tissue-specific promoter or other regulatory sequence and pharmaceutically acceptable formulations including that nucleic acid or biologically active variants thereof) and methods that can be used to effectively modify the content of PUFAs in animal cells (i.e., cells other than those of *C. elegans*, for example, avian or fish cells such as myocytes, neurons (whether of the peripheral or central nervous system), adipocytes, endothelial cells, and cancer cells). The compositions and methods include a fat-1 gene that has been modified to include at least one optimized codon. The modified cells, whether in vivo or ex vivo (e.g., in tissue culture), transgenic animals containing them (fish and birds in particular), and food products obtained from those animals (e.g., meat or other edible parts of the animals (e.g., liver, kidney, or sweetbreads)) are also within the scope of the present invention.

WO 2005/077022 A3